
Development of a human stem cell-derived inhibitory neuron therapeutic for the treatment of chronic focal epilepsy

Grant Award Details

Development of a human stem cell-derived inhibitory neuron therapeutic for the treatment of chronic focal epilepsy

Grant Type: Therapeutic Translational Research Projects

Grant Number: TRAN1-11611

Investigator:

Name:	Cory Nicholas
Institution:	Neurona Therapeutics
Type:	PI

Disease Focus: Epilepsy, Neurological Disorders

Human Stem Cell Use: Embryonic Stem Cell

Award Value: \$4,848,750

Status: Pre-Active

Grant Application Details

Application Title: Development of a human stem cell-derived inhibitory neuron therapeutic for the treatment of chronic focal epilepsy

Public Abstract:**Translational Candidate**

A cellular therapeutic comprised of inhibitory nerve cells produced from human stem cells

Area of Impact

Drug-resistant chronic temporal lobe epilepsy

Mechanism of Action

The product candidate is intended to be delivered into the seizure focus, integrate, and secrete the inhibitory neurotransmitter GABA to rebalance neural electrical activity in the brain and eliminate/reduce seizures.

Unmet Medical Need

The seizures in approximately one-third of epilepsy patients do not adequately respond to current anti-epileptic drugs. Alternative surgical interventions are highly invasive and damage brain tissues. The proposed product candidate is intended to be restorative and long-acting.

Project Objective

Pre-IND meeting; Pilot material manufactured

Major Proposed Activities

- Finalize manufacturing process to be appropriate for future clinical use
- Produce Pilot product using the intended process, confirm efficacy in two rodent models of chronic epilepsy and demonstrate safety at maximum dose
- Select intended clinical cell delivery device and conduct preIND meeting to confirm IND-enabling preclinical requirements

Statement of Benefit to California:

Epilepsy is the fourth most common neurological disorder affecting more than 400,000 people in the State of California. One-third of epilepsy patients are considered to be drug-resistant and have persistent, uncontrolled seizures that can be disabling and affect quality of life. Alternative surgical interventions are highly invasive and may cause lasting impairment. This proposal aims to further develop a cellular therapeutic for treating drug-resistant epilepsy.

Source URL: <https://www.cirm.ca.gov/our-progress/awards/development-human-stem-cell-derived-inhibitory-neuron-therapeutic-treatment>